2018 Current Fiscal Year Report: Cellular Tissue and Gene Therapies Advisory Committee

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1. Department or Agency	2. Fiscal Year
Department of Health and Human Services	2018

3. Committee or Subcommittee	3b. GSA Committee No.
Cellular Tissue and Gene Therapies Advisory Committee	127

4. Is this New During Fiscal Year?	5. Current Charter	6. Expected Renewal Date	7. Expected Term Date
No	10/28/2016	10/28/2018	

8a. Was Terminated During FiscalYear?	8b. Specific Termination Authority	8c. Actual Term Date
No		

9. Agency Recommendation for Next FiscalYear	10a. Legislation Req to Terminate?	10b. Legislation Pending?
Continue	Not Applicable	Not Applicable

11. Establishment Authority Authorized by Law

12. Specific Establishment Authority	13. Effective Date	14. Commitee Type	14c. Presidential?
21 U.S.C. 394	11/28/1990	Continuing	No

15. Description of Committee Scientific Technical Program Advisory Board

16a. Total Number of Reports No Reports for this FiscalYear

17a. Open 1 **17b. Closed** 0 **17c. Partially Closed** 0 **Other Activities** 0 **17d. Total** 1

Meetings and Dates

Purpose	Start	End
On October 12, 2017, the Cellular, Tissue and Gene Therapies Advisory Committee (CTGTAC) met in an open session to discuss and make recommendations on the safety and effectiveness of Biologics License Application (BLA) 125610, voretigene neparvovec, submitted by Spark Therapeutics, Inc. The proposed indication (use) for this product is treatment of patients with vision loss due to confirmed biallelic RPE65 mutation-associated retinal dystrophy.	10/12/2017	10/12/2017

Number of Committee Meetings Listed: 1

	Current FY	Next FY
18a(1). Personnel Pmts to Non-Federal Members	$4,650.00	$44,841.00
18a(2). Personnel Pmts to Federal Members	$0.00	$0.00
18a(3). Personnel Pmts to Federal Staff	$241,098.00	$270,747.00
18a(4). Personnel Pmts to Non-Member Consultants	$0.00	$33,904.00
18b(1). Travel and Per Diem to Non-Federal Members	$9,374.00	$56,632.00
18b(2). Travel and Per Diem to Federal Members	$0.00	$0.00
18b(3). Travel and Per Diem to Federal Staff	$0.00	$0.00

18b(4). Travel and Per Diem to Non-member Consultants	$4,678.00	$28,263.00
18c. Other(rents,user charges, graphics, printing, mail, etc.)	$66,659.00	$106,383.00
18d. Total	$326,459.00	$540,770.00
19. Federal Staff Support Years (FTE)	1.70	1.85

20a. How does the Committee accomplish its purpose?

The Committee reviews and evaluates available data relating to the safety, effectiveness, and appropriate use of human cells, human tissues, gene transfer therapies and xenotransplantation products which are intended for transplantation, implantation, infusion and transfer in the prevention and treatment of a broad spectrum of human diseases and in the reconstruction, repair or replacement of tissues for various conditions. On October 12, 2017, the Cellular, Tissue and Gene Therapies Advisory Committee (CTGTAC) met in an open session to discuss and make recommendations on the safety and effectiveness of Biologics License Application (BLA) 125610, voretigene neparvovec, submitted by Spark Therapeutics, Inc. The proposed indication (use) for this product is treatment of patients with vision loss due to confirmed biallelic RPE65 mutation-associated retinal dystrophy.

20b. How does the Committee balance its membership?

Members have clinical or preclinical and product experience in the fields of cellular therapies, tissue transplantation, gene transfer therapies, and xenotransplantation including biostatistics, bioethics, hematology/oncology, human tissues and transplantation, reproductive medicine, general medicine and various medical specialties including surgery and oncology, immunology, virology, molecular biology, cell biology, developmental biology, tumor biology, biochemistry, rDNA technology, nuclear medicine, gene therapy, infectious diseases, and cellular kinetics. One member is technically qualified and identified with consumer interests and one non-voting member represents the point of view of industry.

20c. How frequent and relevant are the Committee Meetings?

The Committee met one time in FY2018. For FY 2019 the committee anticipates to hold at least 6 open meetings.

20d. Why can't the advice or information this committee provides be obtained elsewhere?

Members of the Committee are drawn from academia, research, and/or clinical practice. Their advice and input lends credibility to regulatory decisions. The alternate means of obtaining this advice would involve the recruitment of large numbers of scientists on a full-time basis at maximum rates of compensation.

20e. Why is it necessary to close and/or partially closed committee meetings?

N/A

21. Remarks

This committee had no reports for FY18.

Designated Federal Officer

Prabhakara Lakshmi Atreya Division of Scientific Advisors and Consultants

Committee Members	Start	End	Occupation	Member Designation
Ando, Dale	04/01/2015	03/31/2019	Gene Editing and Gene Therapy Consulting	Representative Member
Bartlett, David	10/21/2014	03/31/2018	Surgical Oncology, University of Pittsburgh	Special Government Employee (SGE) Member
Berns, Kenneth	09/27/2018	03/31/2022	Distinguished Professor Emeritus, University of Florida	Special Government Employee (SGE) Member
Bollard, Catherine	09/30/2015	03/31/2019	Pediatric oncology, Children's National Medical Center	Special Government Employee (SGE) Member
Breuer, Christopher	06/07/2018	03/31/2022	Co-Director, Nationwide Children's Hospital, Columbus, Ohio	Special Government Employee (SGE) Member
Butterfield, Lisa	11/28/2016	03/31/2020	Professor, Surgery and Immunology, U. Pittsburgh, Pittsburgh, PA	Special Government Employee (SGE) Member
Byrne, Barry	03/20/2014	03/31/2018	Director, Powell Center for Rare Disease Research, Powell Gene Therapy Center, Gainesville, FL	Special Government Employee (SGE) Member
Hawkins, Randy	04/01/2017	03/31/2021	Consumer Representative, Private Practice,Inglewood, CA	Special Government Employee (SGE) Member
Longo, Daniel	04/01/2017	03/31/2021	Hematology, Professor of Medicine, Brigham and Womens Hospital, Boston, MA	Special Government Employee (SGE) Member
Morrison, Sean	06/07/2018	03/31/2022	Director, Children's Research Institute, University of Texas Southwestern Medical Center, Dallas,Texas	Special Government Employee (SGE) Member
Pluhar, Grace	04/01/2014	03/31/2018	Associate Professor, College of Vet Med, University of Minnesota	Special Government Employee (SGE) Member
Roos, Raymond	02/21/2017	03/31/2020	Professor Neurological Sciences, University of Chicago, Chicago, IL	Special Government Employee (SGE) Member
Stegemann, Jan	06/06/2016	03/31/2020	Biomedical Engineer, University of Michigan, Ann Arbor, MI	Special Government Employee (SGE) Member
Walters, Mark	09/27/2018	03/31/2022	Jordan Family Director, USCF Benioff Children's Hospital Oakland, Oakland, CA	Special Government Employee (SGE) Member
Wittes, Janet	11/18/2015	03/31/2019	Mathematics and BioStatistics, Statistical Collaborative, Washington DC	Special Government Employee (SGE) Member
Wu, Joseph	11/28/2016	03/31/2020	Director, Stanford Cardiovascular Inst. and Professor of Medicine and Radilogy, Stanford University, Stanford, CA	Special Government Employee (SGE) Member
Zaia, John	06/07/2018	03/31/2022	Director, Center for Gene Therapy, Beckman Research Institute of City of Hope, Duarte, CA	Special Government Employee (SGE) Member
Zovein, Ann	04/01/2014	03/31/2018	Stem Cell and Devt. Biology, UCSF Cardiovascular Research Institute, San Francisco, CA	Special Government Employee (SGE) Member

Number of Committee Members Listed: 18

Narrative Description

FDA's strategic priorities in responding to the public health challenges of the 21st century

are to advance regulatory science and innovation; strengthen the safety and integrity of the global supply chain; strengthen compliance and enforcement activities to support public health; expand efforts to meet the needs of special populations; advance medical countermeasures and emergency preparedness; advance food safety and nutrition; promote public health by advancing the safety and effectiveness of medical products; establish an effective tabacco regulation, prevention, and control program; and manage for organizational excellence and accountability. The Cellular, Tissue and Gene Therapies Advisory Committee supports FDA's mission and strategic action by reviewing and evaluating available data relating to the safety and effective use of cellular therapies, tissue transplantation, gene transfer therapies and xenotransplantation, which are intended for the use in the prevention and treatment of a broad spectrum of human diseases. The Committee also considers the quality and relevance of FDA's research program, which provides scientific support for the regulation of these products. The Committee supports FDA's mission by using science-based risk management in all of its activities. The Committee recommendations provide the most health promotion and protection at the least cost for the public. This Committee assists the Agency in ensuring timely, high quality, cost-effective processes for review of new technologies/pre-market submissions, effective communication and working relationships with stakeholders to enhance U.S. and global health outcomes, accurately analyzing risks associated with medical products, facilitating the development and availability of medical countermeasures to limit the effects of a terrorist attack on the civilian and military populations, protecting the safety and security of biologics (gene therapy, human tissues, and cellular therapies) all key components of FDA's strategic plan objectives.

What are the most significant program outcomes associated with this committee?

	Checked if Applies
Improvements to health or safety	✓
Trust in government	✓
Major policy changes	✓
Advance in scientific research	✓
Effective grant making	
Improved service delivery	
Increased customer satisfaction	✓
Implementation of laws or regulatory requirements	✓
Other	

Outcome Comments

NA

What are the cost savings associated with this committee?

	Checked if Applies
None	☐
Unable to Determine	☑
Under $100,000	☐
$100,000 - $500,000	☐
$500,001 - $1,000,000	☐
$1,000,001 - $5,000,000	☐
$5,000,001 - $10,000,000	☐
Over $10,000,000	☐
Cost Savings Other	☐

Cost Savings Comments

The utilization of the Cellular, Tissue and Gene Therapies Advisory Committee enables the Agency to obtain required and frequently scarce professional services from medical and scientific experts not otherwise available to the Agency; and to obtain the services of these experts only on an as needed basis rather than on a full time basis. The services of the Committee resulted in advice for the improvement of the public health, for which it is difficult to assign a financial value.

What is the approximate Number of recommendations produced by this committee for the life of the committee?

85

Number of Recommendations Comments

The Committee made 85 recommendations from FY2003 through FY2018. See 20a of the Annual Report for specific accomplishments.

What is the approximate Percentage of these recommendations that have been or will be Fully implemented by the agency?

80%

% of Recommendations Fully Implemented Comments

The function of an advisory committee is purely advisory in nature. Although the FDA most often accepts the recommendations from its committees, the advice is purely advisory in nature, and therefore, the Agency has the option of not implementing the advice.

What is the approximate Percentage of these recommendations that have been or will be Partially implemented by the agency?
9%

% of Recommendations Partially Implemented Comments
The function of an advisory committee is purely advisory in nature. Although the FDA most often accepts the recommendations from its committees, the advice is purely advisory in nature, and therefore, the Agency has the option of not implementing the advice.

Does the agency provide the committee with feedback regarding actions taken to implement recommendations or advice offered?
Yes ☑ No ☐ Not Applicable ☐

Agency Feedback Comments
It usually does. Product approval issues are first released to the sponsor. When appropriate, information is made available to the public. Actions related to guidance documents or other general matters issues are available publicly when implemented.

What other actions has the agency taken as a result of the committee's advice or recommendation?

	Checked if Applies
Reorganized Priorities	☑
Reallocated resources	☐
Issued new regulation	☑
Proposed legislation	☐
Approved grants or other payments	☐
Other	☑

Action Comments
FDA approves or chooses not to approve an investigational medical product.

Is the Committee engaged in the review of applications for grants?
No

Grant Review Comments

NA

How is access provided to the information for the Committee's documentation?

	Checked if Applies
Contact DFO	✓
Online Agency Web Site	✓
Online Committee Web Site	✓
Online GSA FACA Web Site	✓
Publications	✓
Other	

Access Comments

N/A